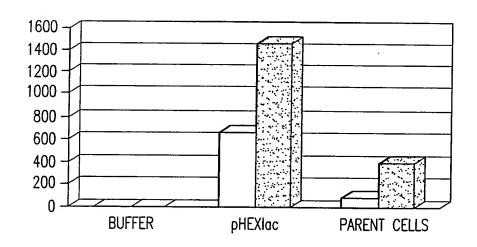


FIG.1A



☐ HEX-A ₩ HEX-A & HEX-B

FIG.1F

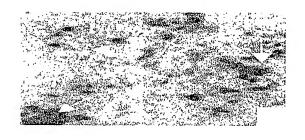


FIG.1B

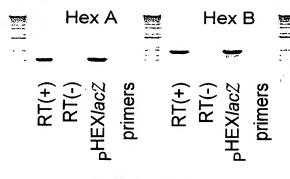


FIG.1C



FIG.1D1



FIG.1D2



FIG.1E1



FIG.1E2

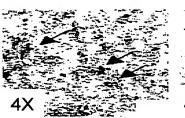
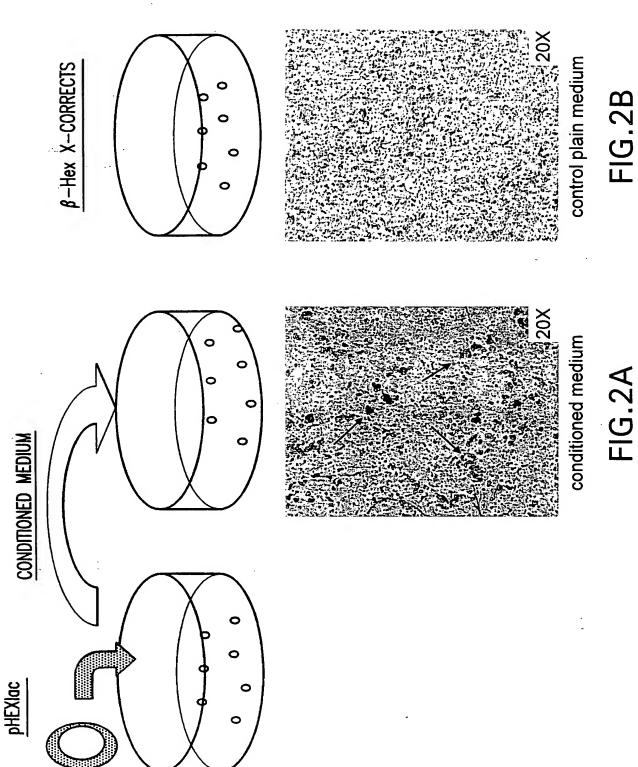


FIG.1G1



FIG.1G2



CMV-HexA-IRES-HexB-pA VSV-G PSEUDOTYPED FIV(Hex) Agag-Apol-VSV-G gag-pol-vif-Aenv Δgag 293 CELLS CMV-R-U5 CMV-R-U5 CMV-R-U5

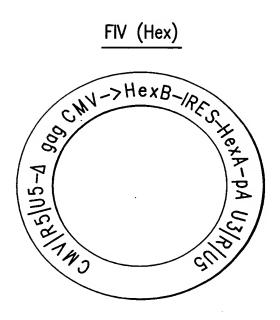


FIG.4

FIV (Hex) 6.2 maxi prep Total 1mg 1kb<sup>+</sup> uncut Sca l Not l Sal l Xho l 1kb<sup>+</sup> (1μg/μl)

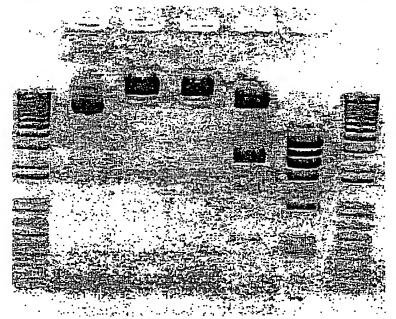


FIG.5

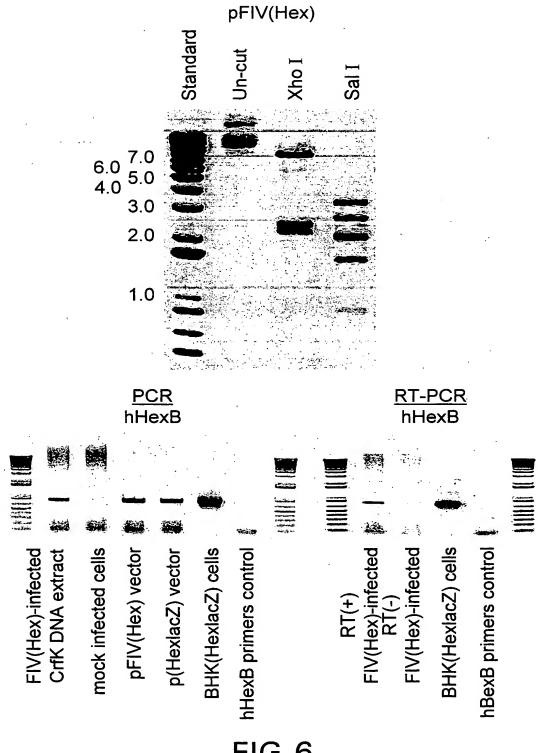


FIG.6

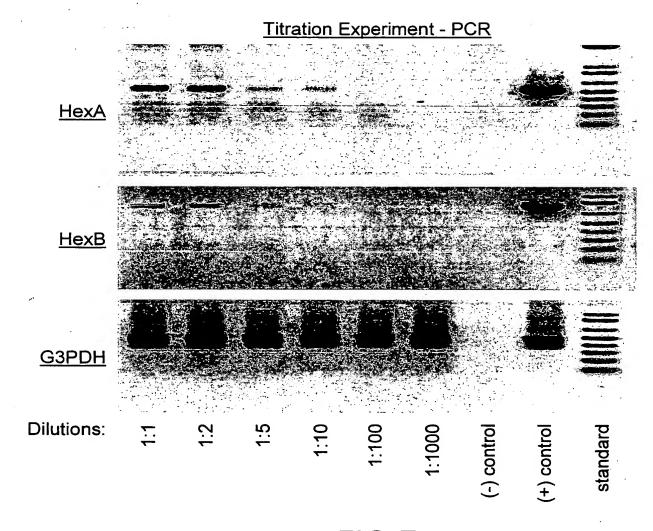


FIG.7

**Ε**.α

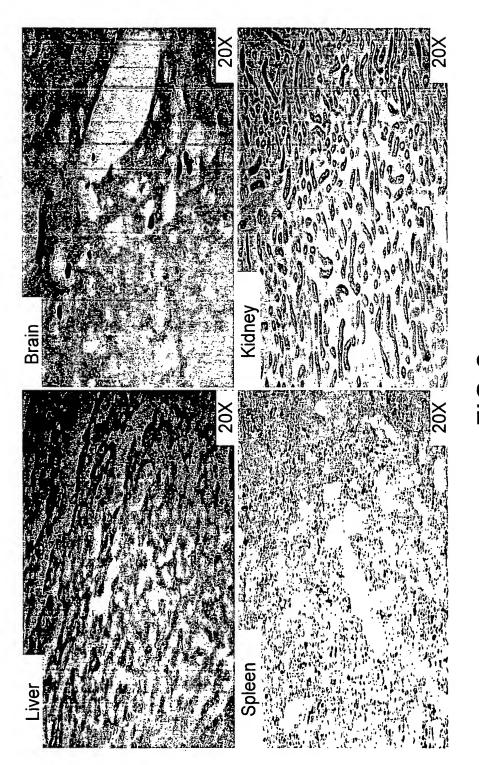
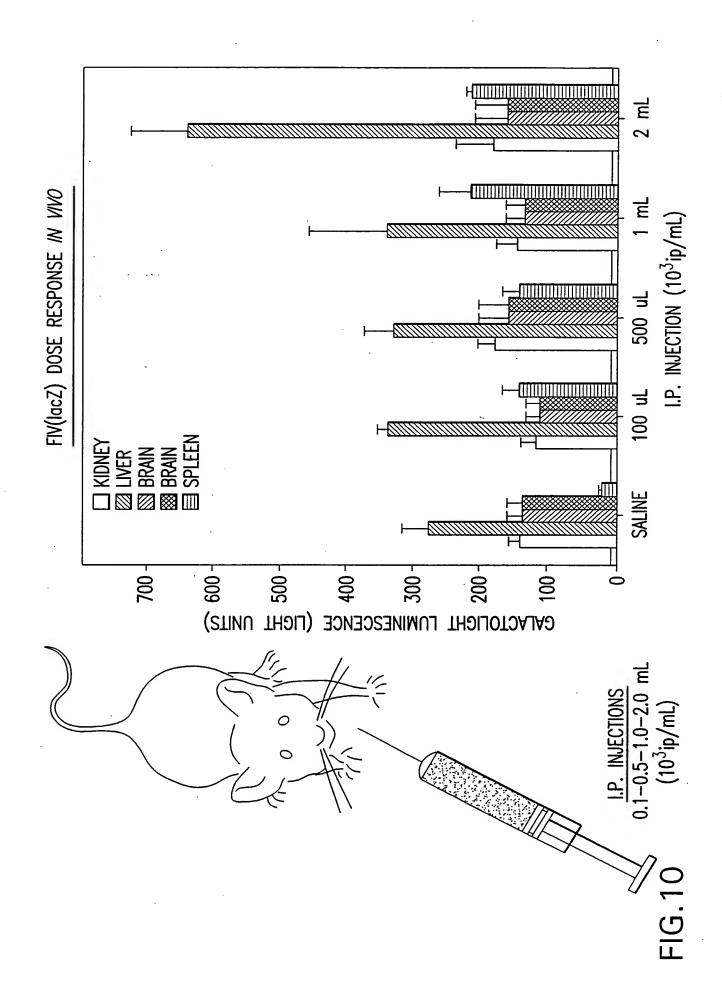
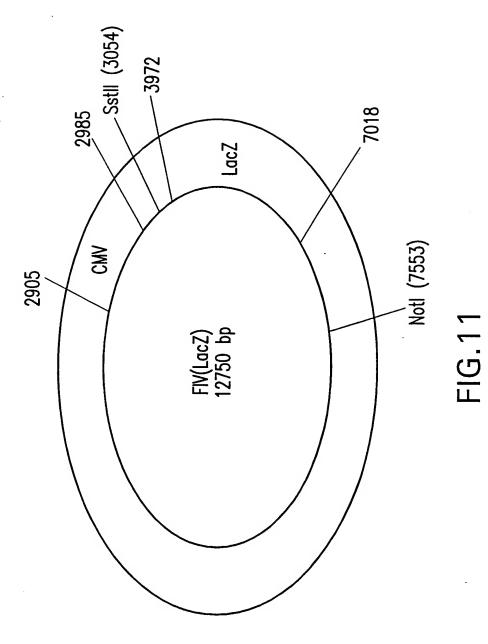


FIG.9





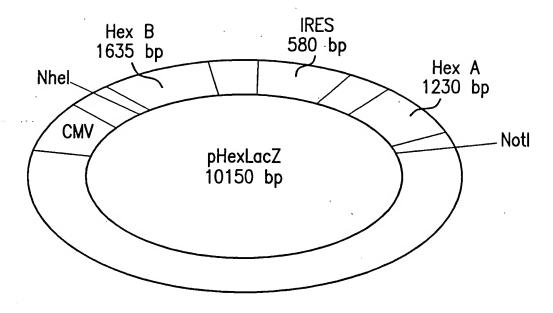


FIG.11A

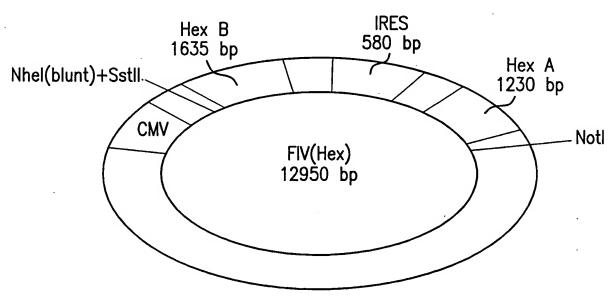
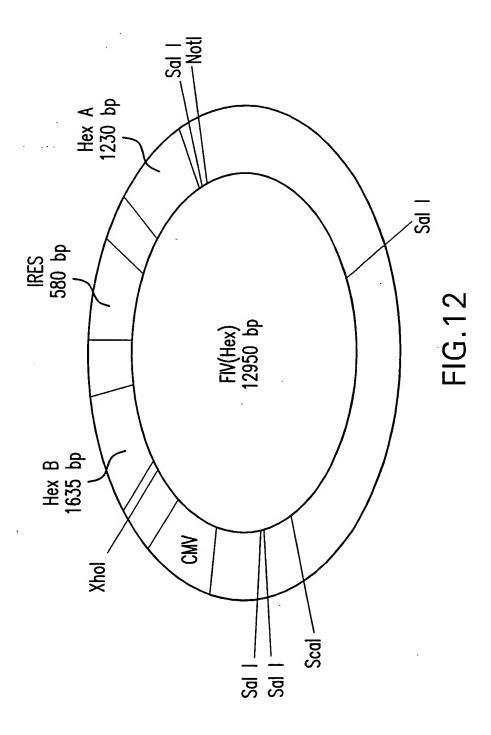
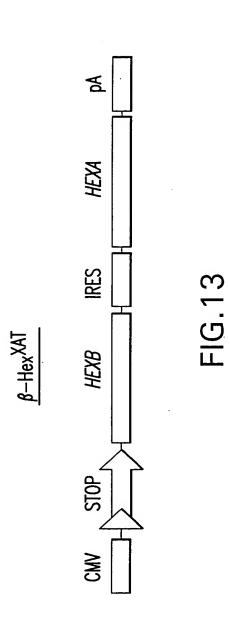


FIG.11B





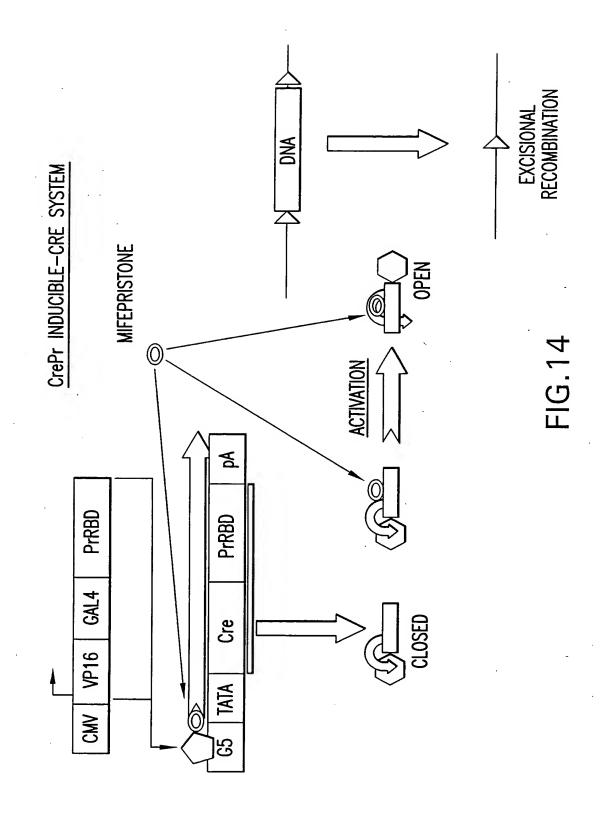
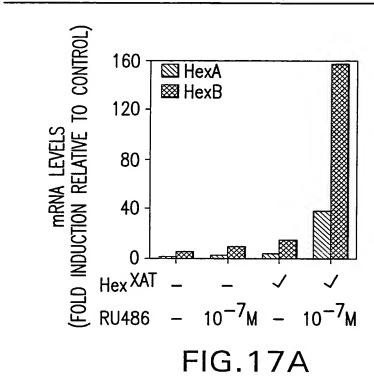


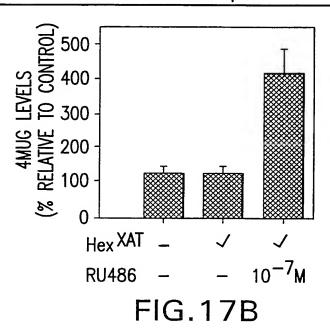
FIG. 15

ဖ S RU486 10<sup>-7</sup> 0 10<sup>-7</sup>  $\mathsf{Hex}^\mathsf{XAT}$ : Excisionally activated  $\beta$ -hexosaminidase gene PARTY. Later Section 1 HexXAT G3PDH GLVP CrePr HexA HexB ]] 9 S FIG.16A pHexXAT  $\rightarrow$  STOP  $\rightarrow$  HexB ഗ Activated Dormant 13

FIG.16C



Hex XAT: EXCISIONALLY ACTIVATED  $\beta$ -HEXOSAMINIDASE GENE



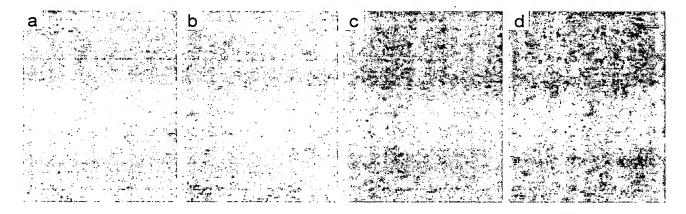


FIG.17C

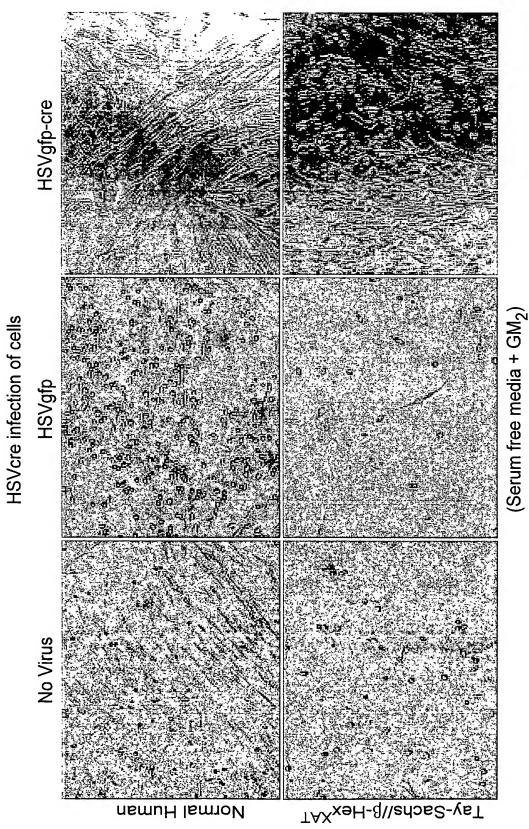


FIG.18

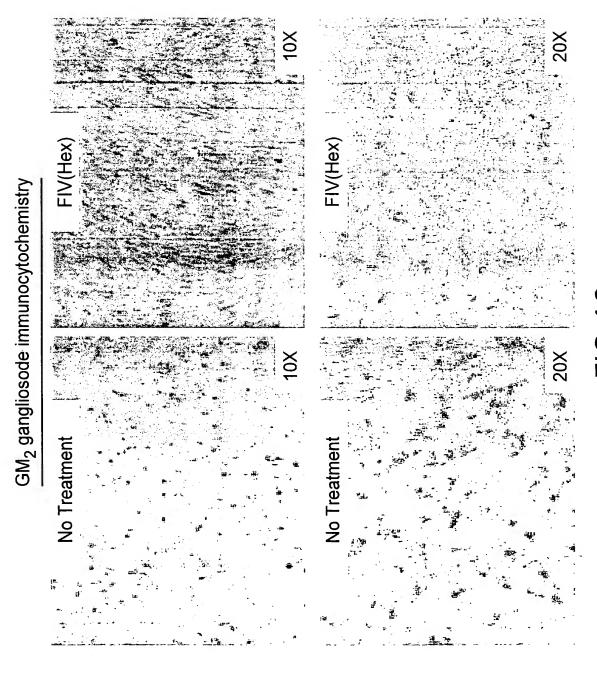
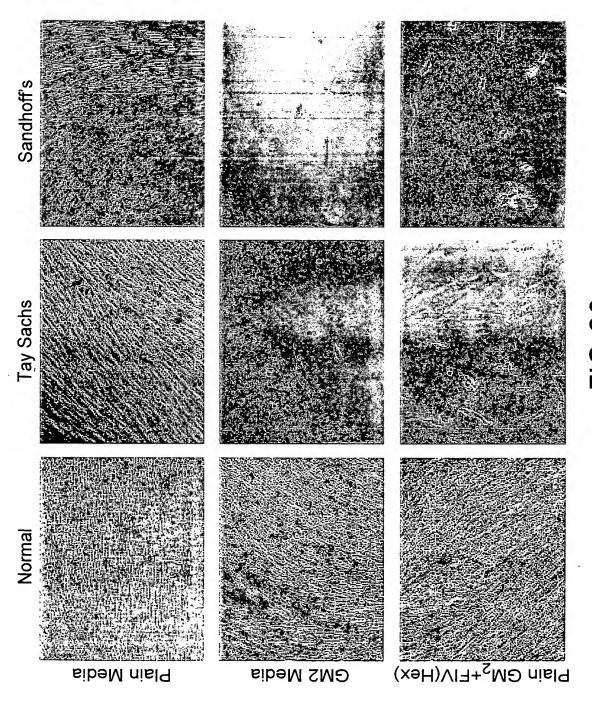


FIG. 19





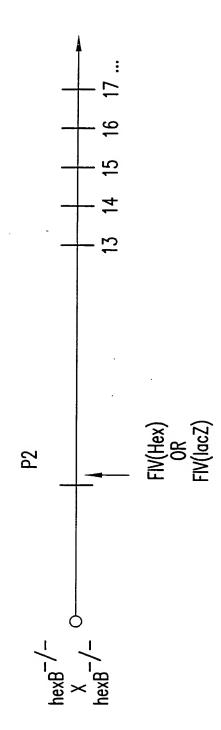


FIG. 21

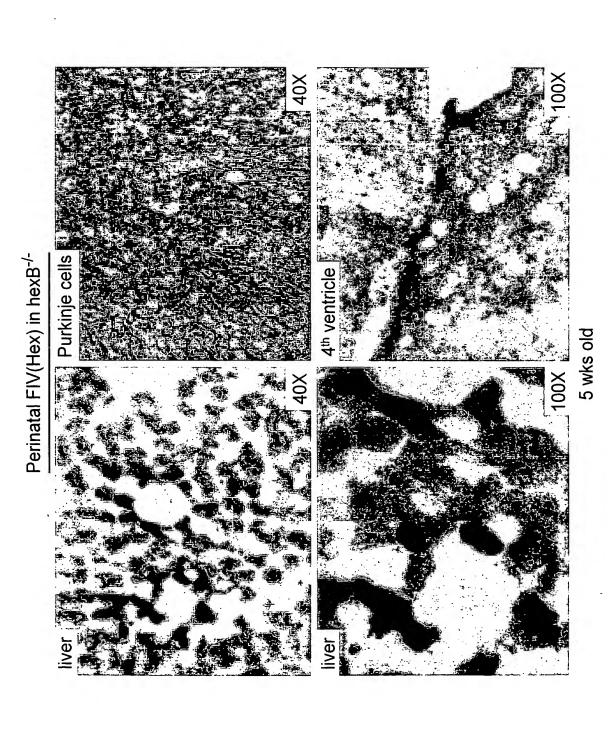


FIG.22

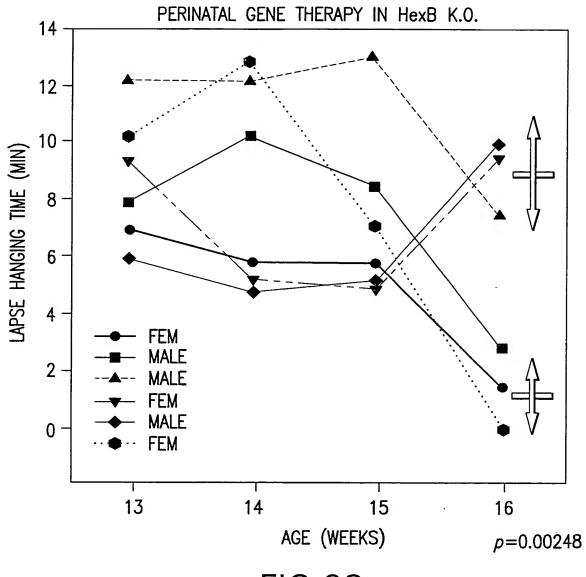


FIG.23

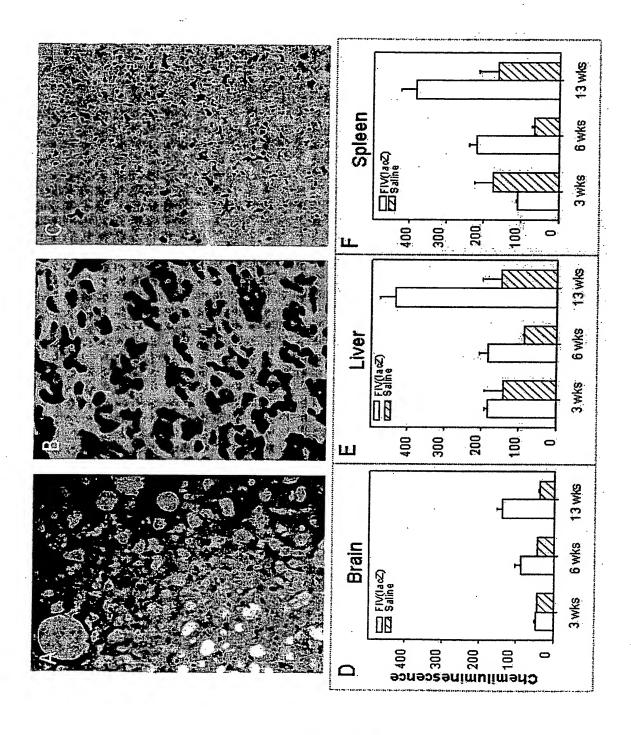


FIG. 24

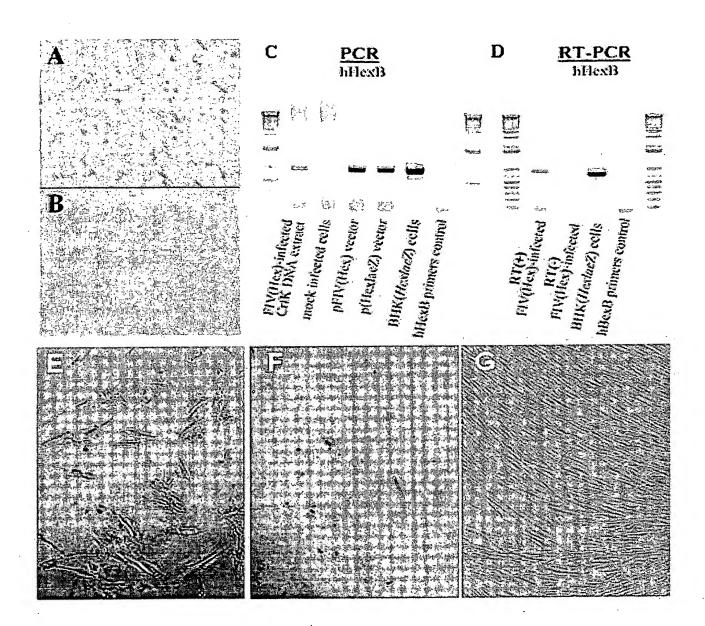


FIG. 25

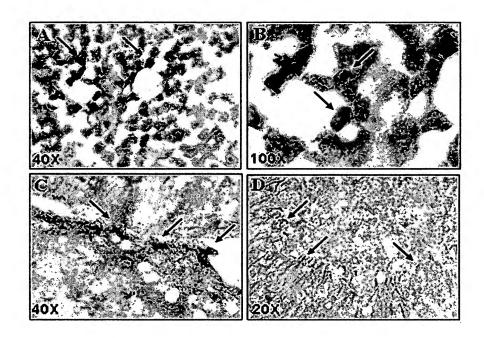


FIG. 26

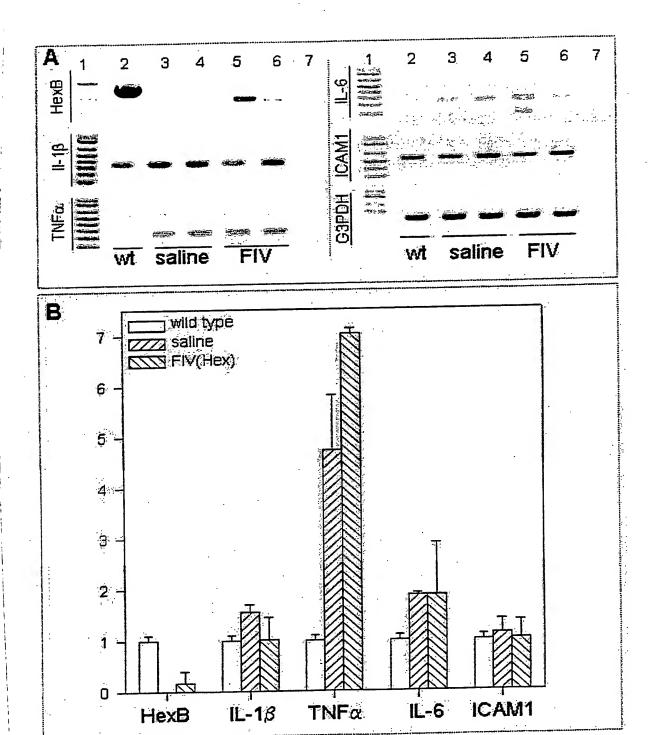


FIG. 27

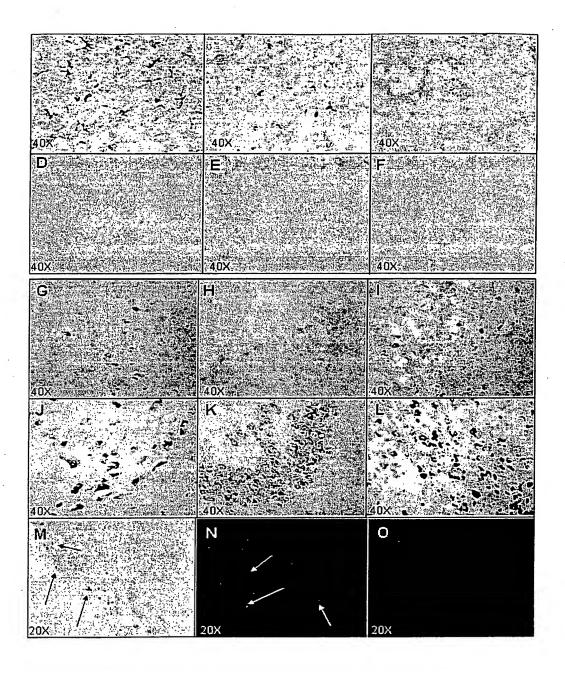


FIG. 28

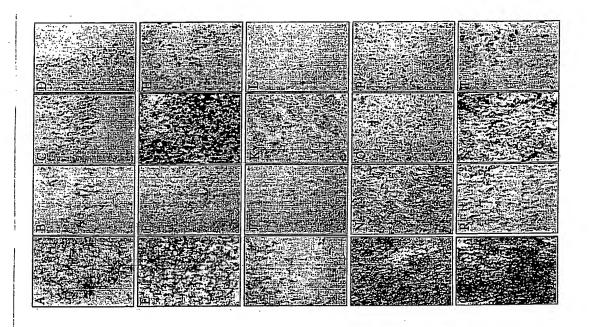


FIG. 29

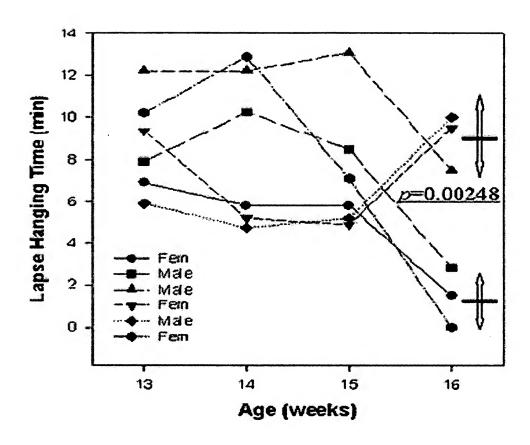
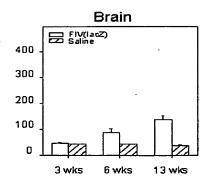
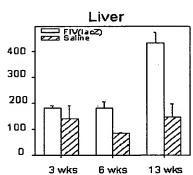


FIG. 30





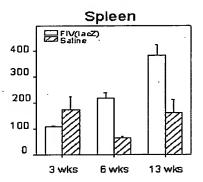


FIG. 31

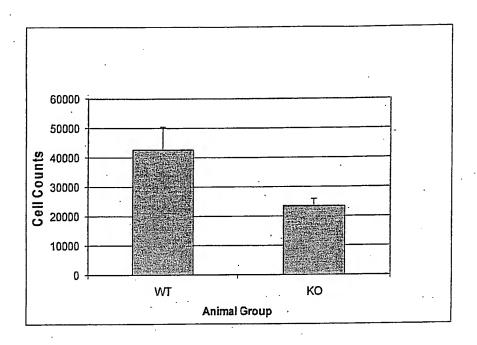


FIG. 32